EXHIBIT A

NEW YORK UNIVERSITY
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Charles Seife, Professor
ARTHUR L. CARTER JOURNALISM INSTITUTE

Department of Health and Human Services
Mary E. Switzer Building, Room 2221
330 C Street, S.W.
Washington, DC 20201

U.S. Food and Drug Administration
5630 Fishers Lane
Room 1035
Rockville, MD 20857

December 5, 2016

RE: EXPEDITED FREEDOM OF INFORMATION ACT REQUEST

To Whom It May Concern:

This is a Freedom of Information Act ("FOIA") request for information regarding the Food and Drug Administration's ("FDA") recent approval of the drug eteplirsen, made by Sarepta Therapeutics ("Sarepta") and marketed as Exondys 51. I am Professor Charles Seife, Professor of Journalism at New York University and acclaimed science and math journalist. I am dedicated to improving transparency in the arena of health and healthcare by disseminating information related to drug and device approval that is of vital public importance.

On September 19, 2016, the FDA approved eteplirsen for Duchenne muscular dystrophy ("Duchenne"), a fatal childhood disease. The approval came on the heels of intense pressure from patient groups, calls from 109 members of Congress,[1] and shareholder concerns that the drug's manufacturer—the only company developing a treatment for Duchenne—would fold if the drug were rejected. But behind the scenes, the FDA was locked in a "civil war" over its approval.[2] By the time of approval, a top official had overridden several recommendations *not* to approve the drug, FDA scientists had resigned, and the FDA Commissioner had called for the retraction of one of the two clinical trials used for approval. Serious questions persist regarding the drug's effectiveness and the integrity of Sarepta's clinical trial implementation, and the controversy has received a deluge of media attention. Despite the massive controversy, on the basis of FDA's approval the vast majority of neurologists are poised to prescribe the $300,000 treatment based on FDA's approval. In light of this currently unfolding story, it is vital that the requested clinical information and correspondences related to eteplirsen's approval process be made public.

DOCUMENT REQUESTS

[1] *See* Jett Foundation, *109 Congressional Representatives Stand With Duchenne* (Feb. 18, 2016), http://jettfoundation.org/blog/109-congressional-representatives-stand-with-duchenne/. (A true and correct copy of this article is annexed as Exhibit A.)
[2] Matthew Herper, *Approving A Muscular Dystrophy Drug Ignites A Civil War At The FDA*, Forbes (Sept. 20, 2016, 9:17 AM), http://www.forbes.com/sites/matthewherper/2016/09/20/approving-a-muscular-dystrophy-drug-ignites-civil-war-at-the-fda/#3577c506353d. (A true and correct copy of this article is annexed as Exhibit B.)

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I seek disclosure of the following records under the Freedom of Information Act ("FOIA"), 5 U.S.C. § 552:

1. The Chronology prepared by Virginia Behr and submitted to the Scientific Dispute Resolution Board regarding eteplirsen ("Behr Chronology").[3]

2. Any e-mails, memos, or other correspondences dated from 1/1/2005 to the present which contains one or more of the following words:

    a. "Sarepta"; or
    b. "Eteplirsen"; or
    c. "AVI-4658"; or
    d. "Drisapersen"; or
    e. "Kyndrisa"; or
    f. "PRO051"; or
    g. "GSK2402968"; or
    h. "DMD"; or
    i. "Duchenne"; or
    j. "Dystrophy"; or
    k. "Exondys"; or
    l. "Dystrophin."

    And is:

    m. To or from Robert Califf; or
    n. To or from Margaret Hamburg; or
    o. To or from Janet Woodcock; or
    p. To or from Richard Moscicki; or
    q. To or from Robert Temple.

3. Any e-mails, memos, or other correspondences from Robert Califf and/or Ellis Unger to editors or publishers of the *Annals of Neurology*.

4. Any documents dated from 1/1/2010 onwards mentioning possible or actual recusal by Richard Moscicki from any of his duties.

[3] *See* Luciana Borio, Scientific Dispute Resolution Appeal Regarding Eteplirsen, *in Center for Drug Evaluation and Research Summary Review* 15, 23 (2016), http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000SumR.pdf. (A true and correct copy of this document is annexed in Exhibit C.)

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5. Any e-mails, memos, or other correspondences to or from Richard Moscicki dated from 1/1/2010 onward that:

    a. Mentions or is addressed to Ed Kaye; or
    b. Mentions or is addressed to a Sarepta employee; or
    c. Mentions or is addressed to a BioMarin employee; or
    d. Mentions Genzyme.

6. The following data regarding Study 201/202:

    a. Clinical Study Reports;
    b. Protocols and protocol amendments;
    c. Statistical Analysis Plans and plan amendments; and
    d. Documents of regulatory communications.

I request that all of these documents be produced in their native electronic formats with any attached metadata included, so long as such electronic files can be opened using standard commercially available software. If the files cannot be produced in this manner, I request that records be produced in an alternative electronic format that is text-searchable. With respect to databases, spreadsheets or similar organized sets of data, I request that the records be produced in .xls or .csv format. *See* 5 U.S.C. § 552(a)(3)(B).

BACKGROUND

This FOIA request seeks access to information regarding clinical testing and accelerated FDA approval of eteplirsen. The drug is intended to treat a subset of patients affected by Duchenne, a neuromuscular disorder caused by mutations of the dystrophin gene.[4] Duchenne causes progressive muscle degeneration and death from respiratory or cardiac failure.[5] Eteplirsen received accelerated approval, which is available for drugs treating "serious or life-threatening diseases" that provide "meaningful" benefit over existing therapies.[6] Sarepta satisfied FDA's standard using a twelve-patient clinical trial and the surrogate endpoint of dystrophin production.

The primary scientific bodies tasked with overseeing the review process concluded that eteplirsen should not be approved. Ronald Farkas, who led the clinical review team that unanimously opposed the drug's approval,

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[4] *See* Ellis Unger, Agency Scientific Dispute – Appeal, *in Center for Drug Evaluation and Research Summary Review* 42, 43 (2016) http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000SumR.pdf. (A true and correct copy of this document is annexed in Exhibit C.)

[5] *Id.*

[6] 21 C.F.R. 314(h). There are two paths to accelerated approval: clinical benefit and surrogate endpoints. A drug can thus be approved "on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely . . . to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity." *Id.*

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expressed "strong doubts" about the viability of the clinical trial results.[7] Shortly before eteplirsen's approval, Farkas resigned from the agency.[8] Further, in April 2016 the FDA convened an advisory committee, which voted that there was no evidence of clinical effectiveness and that the drug did not produce dystrophin at a level reasonably likely to result in clinical benefit.[9] The advisory committee hearing illustrated the intense public advocacy surrounding the drug, as audience members "broke into angry shouts" following the final vote.[10]

Controversy reached a boiling point after the recommendations not to approve the drug. Dr. Janet Woodcock, director of FDA's Center for Drug Evaluation and Research ("CDER"), overrode reviewers' objections and granted eteplirsen accelerated approval.[11] Woodcock reached her conclusion based on the drug's effect on the surrogate endpoint of dystrophin production, which she concluded was reasonably likely to predict clinical benefit.[12] However, Dr. Ellis Unger, director of CDER's Office of Drug Evaluation, disagreed with Woodcock's conclusion.[13] Unger's appeal sent the issue to Dr. Robert Califf, the FDA Commissioner, who deferred to Woodcock.[14]

These highly publicized FDA disputes stem principally from Sarepta's clinical trials. Flaws in the design and implementation of these trials "made it impossible to use much of the resulting trial data as reliable evidence in

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[7] Farkas led the clinical team in the neurology products division and made his statements at an advisory committee meeting. Ed Silverman, *FDA Confirms That Critic of Sarepta Drug Has Left the Agency*, STAT (Sept. 14, 2016), https://www.statnews.com/pharmalot/2016/09/14/fda-sarepta-farkas-duchenne/. (A true and correct copy of this article is annexed as Exhibit D.) This team unanimously opposed approval. *See* Ellis Unger, Office of Drug Evaluation-I: Decisional Memo, *in Center for Drug Evaluation and Research Summary Review* 84, 123 (2016), http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000SumR.pdf. (A true and correct copy of this document is annexed in Exhibit C.)

[8] *See* Silverman, *supra* note 7.

[9] Aaron S. Kesselheim & Jerry Avorn, *Approving a Problematic Muscular Dystrophy Drug: Implications for FDA Policy*, JAMA (Oct. 24, 2016), http://jamanetwork.com/journals/jama/fullarticle/2572614. (A true and correct copy of this article is annexed as Exhibit E.)

[10] Marie Powers, *Sarepta Goes Down in Adcom, With Losers All Around*, Bioworld (Apr. 22, 2016), http://www.bioworld.com/content/sarepta-goes-down-adcom-losers-all-around-0. (A true and correct copy of this article is annexed as Exhibit F.)

[11] Janet Woodcock, Center Director Decisional Memo, *in Center for Drug Evaluation and Research Summary Review* 69, 69 (2016), http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000SumR.pdf (A true and correct copy of this document is annexed in Exhibit C.); Zachary Brennan, *Sarepta Wins Controversial FDA Approval for First DMD Drug*, Reg. Aff. Prof. Soc'y (Sept. 19, 2016), http://www.raps.org/Regulatory-Focus/News/2016/09/19/25870/Sarepta-Wins-Controversial-FDA-Approval-for-First-DMD-Drug/. (A true and correct copy of this article is annexed as Exhibit G.)

[12] Woodcock, *supra* note 11.

[13] Unger, *supra* note 4.

[14] Letter from Robert Califf to Janet Woodcock et al. (Sept. 16, 2016), *in Center for Drug Evaluation and Research Summary Review* 2, 2 (2016), http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000SumR.pdf (A true and correct copy of this document is annexed in Exhibit C.); *see, e.g.*, Sabrina Tavernise, *F.D.A. Approves Muscular Dystrophy Drug That Patients Lobbied For*, N.Y. Times. (Sept 9, 2016), http://nyti.ms/2cXFPQh. (A true and correct copy of this article is annexed as Exhibit H.)

NEW YORK UNIVERSITY
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Charles Seife, Professor
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regulatory decision-making."[15] The original trial, Study 201, used only twelve patients, and after only 24 weeks the control group was removed.[16] And it appears that there were several significant changes not just to the protocol but to the endpoints of the trial after the study was underway, including extending the trial from 24 to 28 weeks,[17] eliminating a key secondary efficacy endpoint,[18] and generating a new secondary endpoint.[19] Further, a dramatic mismatch between two methods of measuring the concentration of dystrophin[20] (along with an equally striking mismatch between the study sponsors' readings of the values versus blinded pathologists' readings),[21] the failure to have a pre-specified statistical analysis plan,[22] and the proposed use of historical controls in a "statistically uninterpretable" manner[23] all raise serious questions about the quality and integrity of Sarepta's generation and communication of study outcomes.

Almost as soon as eteplirsen was approved, multiple FDA officials—including the Commissioner himself—called for this study to be retracted because it was misleading.[24] However, Sarepta had already reported the results of this continuous Study 201/202 in a press release as a remarkable treatment, creating unrealistic expectations in the affected patient community.[25] Even if Sarepta's studies were taken at face value, it is unclear, as Unger suggests, whether the results of Study 201/202, and the phase III trial 301 meet the diminished standard of evidence required for accelerated approval; specifically, the degree of increased dystrophin production in the trial appears to be an order of magnitude below the amount that is reasonably likely to predict clinical benefit, as suggested by the amount of dystrophin that is cited to be important in affecting the course of patients with Becker muscular dystrophy (a less severe form of muscular dystrophy).[26]

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[15] Letter from Robert Califf to Janet Woodcock et al., *supra* note 21 at 18.
[16] *Id.* at 17.
[17] *See* Xiang Ling et al., Statistical Review for NDA206488, *in Peripheral and Central Nervous System Drugs Advisory Committee Meeting* 95, 100 (2016), http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/PeripheralandCentralNervousSystemDrugsAdvisoryCommittee/UCM497063.pdf. (A true and correct copy of this document is annexed in Exhibit I.)
[18] *See* View of NCT01396239 (July 15, 2011), https://clinicaltrials.gov/archive/NCT01396239/2011_07_15. (A true and correct copy of this article is annexed as Exhibit J.) *Cf.* View of NCT01396239 (Nov. 6, 2015), https://clinicaltrials.gov/archive/NCT01396239/2015_11_06. (A true and correct copy of this document is annexed as Exhibit K.)
[19] Ling, *supra* note 17.
[20] Unger, *supra* note 7 at 103.
[21] *Id.* at 97.
[22] Ling, *supra* note 17 at 99.
[23] *Id.*
[24] Ed Silverman, *FDA Commissioner Calls for Sarepta Drug Study to be Retracted,* STAT (Sept. 22, 2016), https://www.statnews.com/pharmalot/2016/09/22/fda-sarepta-duchenne-study-retraction/. (A true and correct copy of this article is annexed as Exhibit L.)
[25] Letter from Robert Califf to Janet Woodcock et al., *supra* note 14 at 22-23.
[26] Unger, *supra* note 4 at 58.

NEW YORK UNIVERSITY
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At least one national health-insurance firm, Anthem, has said it will not pay for the drug, pointing to the incomplete trial data used to approve it.[27] Still, 70 percent of surveyed neurologists are fairly convinced that the drug is sufficiently effective,[28] so many affected boys will likely begin using it, at the cost of $300,000, without much evidence of its efficacy. As Unger explained, "[b]y allowing the marketing of an ineffective drug, essentially a scientifically elegant placebo, thousands of patients and their families would be given false hope in exchange for hardship and risk."[29]

Given the tenuous evidence of effectiveness and the steep price, the public should have full access to information relating to eteplirsen's approval.

FEE WAIVER REQUEST

A waiver of search and review fees is appropriate here, because disclosure of the requested information is in the public interest under the meaning of 5 U.S.C. § 552(a)(4)(A)(ii)(III) and 45 C.F.R. §§ 5.45(a)(1), (b); and because I do not have any commercial interest in disclosure, 45 C.F.R. §§ 5.45(a)(2), (c).

Disclosure Is in the Public Interest

Disclosure of the requested information is likely to contribute significantly to public understanding of the FDA's operations and activities. 45 C.F.R. § 5.45(b)(1). Specifically, the requested information will "reveal meaningful information" that is "not already public knowledge" about the FDA's accelerated drug approval process and the quality of its decision-making. 45 C.F.R. § 5.45(b)(2).

Disclosure is especially important given the high degree of public skepticism about the efficacy of eteplirsen and the processes behind its approval. Access to correspondences between the FDA and Sarepta will likely allow the public to ensure that the FDA did not bow to external pressures when it approved eteplirsen. It will also allow the public to judge whether the standards for accelerated approval—and the use of surrogate endpoints—are adequate to ensure that the agency properly gauges the effectiveness of a drug. And access to information related to the accelerated approval is necessary for the public to independently evaluate whether the FDA was adhering to its own public safety standards when it approved eteplirsen and whether the high price of the drug is justified given the circumstances surrounding eteplirsen's approval.

The public also has an interest in understanding why Sarepta designed and executed Study 201/202 in the way that it did: why it made significant changes to protocols and endpoints while the trial was underway, why it

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[27] Don Seiffert, *Health Insurers Split on Coverage of Sarepta's Duchenne Drug,* Boston Business Journal (Oct. 7, 2016, 2:58 PM) http://www.bizjournals.com/boston/blog/bioflash/2016/10/health-insurers-split-on-coverage-of-sarepta-s.html. (A true and correct copy of this article is annexed as Exhibit M.)

[28] *See* Ed Silverman, *Doctors Expect to Prescribe Sarepta's DMD Drug Despite Insurance Concerns*, STAT (Sept. 27, 2016), https://www.statnews.com/pharmalot/2016/09/27/doctors-sarepta-insurance-coverage-worries/. (A true and correct copy of this article is annexed as Exhibit N.)

[29] Unger, *supra* note 4 at 62.

NEW YORK UNIVERSITY
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Charles Seife, Professor
ARTHUR L. CARTER JOURNALISM INSTITUTE

failed to create a statistical analysis plan and used dubious comparator groups to gauge efficacy, and how the company reacted to the revelation that its measurements of dystrophin were inconsistent. This unusual approval process raises concerns that Sarepta may have intentionally misled the FDA in order to facilitate approval of eteplirsen despite its questionable efficacy. Access to correspondences between Sarepta and the FDA, together with access to information regarding Sarepta's NDA and other records related to the clinical trials, will shed much-needed light on Sarepta's behind-the-scenes conduct and will therefore allow the public to ensure that there was no wrongdoing on Sarepta's part as its $300,000 drug is about to hit the market.

Here, I am "in a position to contribute to public understanding." 45 C.F.R. § 5.45(b)(3). I have covered drug approval for *Scientific American* and *ProPublica* and thus "ha[ve] such knowledge or expertise as may be necessary to understand the information." *Id*. Among other things, I have written on the FDA response to fraudulent research and how pharmaceutical money affects drug research. As a "representative of the news media," my "intended use of the information"—publication—"would be likely to disseminate the information among the public." *Id*. I intend to publish my findings in order to "advance the understanding of the general public." *Id*.

The information I am requesting is "not already public knowledge" under 45 C.F.R. § 5.45(b)(2), because I am specifically requesting the communications that are not currently public in order to substantially advance "the public's understanding of the government's operations" beyond what is in the public record. 45 C.F.R. § 5.45(b)(4). Disclosure of the requested information would make valuable knowledge available to interested scientists. There is a growing consensus in the medical community about the importance of open access to clinical trial data for the advancement of science and the public health.[30] Moreover, public access to the clinical trial data submitted to the FDA in support of drugs that are ultimately approved is crucial in order to permit scientists, physicians, public health professionals, and others to determine whether the FDA is properly discharging its core mission of determining, in a timely fashion, whether pharmaceuticals are safe and effective. If the data—which is not routinely made public—is not released on an expedited basis, experts are unable to independently assess and verify the FDA's safety and efficacy determinations before the drug becomes widely used, which undermines the purpose of independent scrutiny.[31]

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[30] *See, e.g.*, Joseph S. Ross & Harlan M. Krumholz, *Ushering in a New Era of Open Science Through Data Sharing: The Wall Must Come Down*, 309 JAMA no. 13 (Apr. 3, 2013), http://jamanetwork.com/journals/jama/fullarticle/1668313 (A true and correct copy of this article is annexed as Exhibit O.); Ben Goldacre & Carl Heneghan, *Improving, and Auditing, Access to Clinical Trial Results*, BMJ (Jan. 15, 2014), http://dx.doi.org/10.1136/bmj.g213 (A true and correct copy of this article is annexed as Exhibit P.). *Cf*. AllTrials.net, http://www.alltrials.net/ (last visited Nov. 3, 2016).

[31] *See, e.g.*, Harlan M. Krumholz & Eric D. Peterson, Editorial, *Open Access to Clinical Trials Data*, 312 JAMA no. 10 (Sept 10, 2014), http://jamanetwork.com/journals/jama/fullarticle/1902214 (A true and correct copy of this article is annexed as Exhibit Q.); Daniel M. Hartung et al., *Reporting Discrepancies Between the ClinicalTrials.gov Results Database and Peer-Reviewed Publications*, 160 Annals Internal Med. 7 (Apr. 1, 2014) 477, https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4617780/ (A true and correct copy of this article is annexed as Exhibit R.); Peter Lurie & Allison Zieve, *Sometimes the Silence Can Be Like the Thunder: Access to Pharmaceutical Data at the FDA*, 69 Law & Contemp. Probs. 85 (2006) (A true and correct copy of this article is annexed as Exhibit S.); Kristin Rising, Peter Bacchetti & Lisa Bero, *Reporting Bias in Drug Trials Submitted to the Food and Drug Administration: Review of Publication*

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ARTS & SCIENCE

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No Commercial Interest in the Information Sought

The disclosure would not further a commercial interest of the requester. I am making this request the "interest of a representative of the news media in using the information for news dissemination purposes" and therefore do not assert a commercial interest. 45 C.F.R. § 5.45(c)(1). Instead, I aim to further public understanding of the accelerated approval process in furtherance of public knowledge and public health.

For these reasons, a public interest waiver of fees is appropriate here. I therefore respectfully request that all fees related to the search, review, and duplication of the requested records be waived. If the search and review fees will not be waived, I ask that you contact me at the email address listed below should the estimated fees resulting from this request exceed $100.

LIMITATION OF FEES

I am also entitled to a limitation of fees because I am a "representative of the news media" making a request that "is not for a commercial use." 45 C.F.R. § 5.41(b); 5 U.S.C. § 552(a)(4)(A)(ii)(II). As noted above, I am a writer for the New York University Department of Journalism and have written numerous articles on drug research and approval. Moreover, this request is not for commercial use. *See supra*. Thus, in the event that my application for a public interest waiver of all fees is denied, I am nevertheless entitled to a limitation of fees. Specifically, I can only be charged "reasonable standard charges for document duplication," and may not be charged search fees or any other fees. 5 U.S.C. § 552(a)(4)(A)(ii)(II); 45 C.F.R. § 5.41(b).

REQUEST FOR EXPEDITED PROCESSING

I also ask that the information requested be disclosed on an expedited basis. Expedited processing is appropriate here because "a compelling need" exists for the disclosure of the requested information. 5 U.S.C. § 552(a)(6)(E)(i)(I). A compelling need exists when "[w]ith respect to a request made by a person primarily engaged in disseminating information, there is an urgency to inform the public concerning actual or alleged Federal Government activity." 21 C.F.R. § 20.44(a).

As a member of the news media, I am primarily engaged in disseminating information. *Id*. There is also an urgent demand to inform the public about FDA's accelerated approval of eteplirsen. 21 C.F.R. § 20.44(a). The information requested concerns "a matter of exigency to the American public" and "the consequences of delaying a response would compromise a significant recognized interest." *Bloomberg, L.P. v. United States Food & Drug Admin*., 500 F. Supp. 2d 371, 377 (S.D.N.Y. 2007) (quoting *Al-Fayed v. C.I.A*., 254 F.3d 300, 310 (D.C. Cir. 2001)).

The approval of eteplirsen is a matter of exigency to the general American public. Prompt disclosure of the

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and Presentation, Pub. Libr. Sci. Med. (Nov. 25, 2008), http://dx.doi.org/10.1371/journal.pmed.0050217. (A true and correct copy of this article is annexed as Exhibit T.)

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Charles Seife, Professor
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requested information is critical in order to permit the public to understand this controversial approval process. Given that many of FDA's own scientists have raised doubts about the efficacy of this drug, it is important that the public is able to ensure that FDA has not sacrificed public health standards during the approval process *before* the drug enters the market. Given the unconventional aspects of Sarepta's clinical trials, prompt disclosure is also necessary for the public to verify that Sarepta has not committed egregious methodological errors in the implementation of its clinical trials. The requested information is also especially exigent because of the high cost: the public should be able to first independently verify that eteplirsen is safe and effective before this $300,000 drug becomes widely prescribed and used.[32] Furthermore, the approval is exigent because it is "the subject of a currently unfolding story." *Al-Fayed,* 254 F.3d at 310. Since FDA's approval of the drug on September 19, 2016, this decision has garnered a critical amount of media attention: *Forbes*, *The New York Times*, *STAT News*, *The Washington Post*, and many other news media have reported on the matter.[33] A search on the Lexis Nexis database on November 20, 2016 identified 2,272 articles published in 2016 regarding eteplirsen.[34]

Delaying a response would compromise the public's interest in transparency regarding eteplirsen's accelerated approval. According to experts, the use of accelerated approval for eteplirsen moves away from traditional approval processes.[35] Since the accelerated approval for eteplirsen might indicate how drug approval will be conducted in the future,[36] the public's vested interest in understanding the FDA's approval process would be compromised if FDA's response is delayed. Furthermore, while the evidence regarding eteplirsen's effectiveness is tenuous, eteplirsen will probably be used widely once it enters the market. It is therefore important that information on the integrity of its approval process reaches the public *before* the drug becomes widely used. Since eteplirsen also comes with a hefty $300,000 price tag, it is especially important that response to this request is not delayed.

Additionally, there is an urgent need for the information requested because the accelerated approval of eteplirsen might have troubling precedential effect. It provides "a worrisome model for the next generation of molecularly targeted therapies: demonstrate a slight difference in a laboratory test, activate the patient community, win approval, and charge high prices, while relying on limited regulatory follow-up."[37]

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[32] *See supra* note 29.
[33] *See supra* notes 2, 7, 14, 25, 29; Carolyn Y. Johnson, *FDA grants accelerated approval to controversial muscular dystrophy drug*, Wash. Post (Sept. 19, 2016), https://www.washingtonpost.com/news/wonk/wp/2016/09/19/fda-grants-accelerated-approval-to-controversial-muscular-dystrophy-drug/. (A true and correct copy of this article is annexed as Exhibit U.)
[34] Lexis Advance Research, Results for: Eteplirsen, Lexis Nexis (Nov. 20, 2016). Extensive coverage has been cited as a reason for granting expedited processing, as in *Am. Civil Liberties Union of N. California v. U.S. Dep't of Def.*, 2006 WL 1469418 (N.D. Cal. May 205, 2006) (unpublished) at *6 ("There had been at least fifty-three separate articles on the TALON program in the fifty-two days immediately prior to the FOIA requests."). However, such extensive coverage is not necessary, as in *Bloomberg*, 500 F. Supp. 2d at 378 (granting expedited processing even though the record before the agency cited only one relevant May 14, 2006 article and the plaintiff made an initial FOIA request in February 2006 and appealed the constructive denial of the request in April 2006).
[35] *See supra* note 7.
[36] *Id.*
[37] *Id.*

NEW YORK UNIVERSITY
ARTS & SCIENCE

Charles Seife, Professor
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The approval also contains great implications for the 21st Century Cures Act. The 21st Century Cures Act is aimed at, among other things, changing the way FDA approves drugs and medical devices, and it promises to incorporate more of the patient experience into the FDA approval process for medical products.[38] Patient experience played a pivotal role in the process leading up to the approval of eteplirsen;[39] therefore, transparency regarding FDA's accelerated process for eteplirsen would shed light on this Act, which has already been passed by the House and is currently before the Senate.[40] GOP leaders have expressed interest in passing this Act during the lame-duck session,[41] and after the election Senate Majority Leader Mitch McConnell continued to describe the legislation as a top Senate priority for the lame-duck session.[42] Thus, expedited processing is necessary for the public to engage more knowledgably with their Senators regarding this Act, before the Senate potentially passes it. And even if this Act is not passed during the lame-duck session, it is likely that some elements of the Act will be incorporated into the 2017 reauthorization of the Prescription Drug User Fee Act (PDUFA).[43] It is therefore likely that the need for information regarding lowered drug approval standards will continue to remain urgent for years. The requested information therefore "has a particular value that will be lost if not obtained and disseminated quickly." 21 C.F.R. § 20.44(c)(2).

Eteplirsen's approval process is a "breaking news story of general public interest," which is especially evident given the widespread recent coverage of the approval controversy within the FDA.[44] Since FDA's approval of eteplirsen is the subject of a currently unfolding story, it is essential that that I receive this information on an expedited basis so that I can quickly begin working with the disclosed information. *See Al-Fayed*, 254 F.3d at 310. A failure to receive the disclosed information in an expedited manner would seriously harm my ability to inform the public about significant aspects of government activities and would irreparably compromise the public's ability to engage with these matters at this critical time. *Id*. I therefore ask that you grant this request on an expedited basis.

In compliance with 21 C.F.R. § 20.44(a), I certify that the above information pertaining to my request for expedited processing is true and correct to the best of our knowledge and belief.

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[38] *See* Judy Stone, *21st Century Cures Act: Pork Or Promise?*, Forbes (Sept. 22, 2016, 6:30 AM), http://www.forbes.com/sites/judystone/2016/09/22/21st-century-cures-act-pork-or-promise/#51a116986818. (A true and correct copy of this article is annexed as Exhibit V.)
[39] *See* Richard Harris, *Controversy Continues Over Muscular Dystrophy Drug, Despite FDA Approval*, NPR (Sept. 24, 2016), http://www.npr.org/sections/health-shots/2016/09/24/495174472/controversy-continues-over-muscular-dystrophy-drug-despite-fda-approval. (A true and correct copy of this article is annexed as Exhibit W.)
[40] *See supra* note 41.
[41] *See* KHN Morning Briefing, *GOP Lawmakers Enthusiastic About Passing 21st Century Cures Bill In Lame Duck Session*, Kaiser Health News (Oct. 7, 2016), http://khn.org/NjY1NDYx. (A true and correct copy of this article is annexed as Exhibit X.)
[42] *See* David Lim, *McConnell: 21st Century Cures Still Top Priority in Lame Duck*, InsideHealthPolicy.com's Daily Brief (Nov. 10, 2016). (A true and correct copy of this article is annexed as Exhibit Y.)
[43] *See* Todd Allen Wilson, *Inside Health Policy – Industry Looks at 21st Century Cures To Set Stage For PDUFA VI*, Friends of Cancer Research (Dec. 19, 2014). (A true and correct copy of this article is annexed as Exhibit Z.)
[44] *See Wadelton v. Dep't of State*, 941 F. Supp. 2d 120, 123 (D.D.C. 2013).

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Charles Seife, Professor
ARTHUR L. CARTER JOURNALISM INSTITUTE

REQUEST FOR EXPLANATION OF WITHHOLDINGS AND REDACTIONS

If this FOIA request is denied in whole or in part, please provide a reasonable description of any withheld materials and a justification for all such withholdings that includes reference to the specific exemptions of FOIA authorizing withholding and specific reasons why such exemptions apply. 45 C.F.R. § 5.33(a). Under the 2016 FOIA amendments, an agency can withhold information only if "the agency reasonably foresees that disclosure would harm an interest protected by an exemption" or "disclosure is prohibited by law." 5 U.S.C. § 552(a)(8)(A). Therefore, I request that the FDA provide specific reasons why disclosure would harm any interests protected by an exemption. In addition, the new amendment provide that agencies shall "consider whether partial disclosure of information is possible whenever the agency determines that a full disclosure of a requested record is not possible" and shall "take reasonable steps necessary to segregate and release nonexempt information." *Id.* I therefore request that you please release all segregable portions of otherwise exempt material.

FDA cannot reasonably foresee that disclosure of the requested information would harm an interest protected by an exemption. 5 U.S.C. § 552(a)(8)(A). It is unlikely that FDA can show that the requested information would reveal "[t]rade secrets and commercial or financial information obtained from a person and privileged or confidential" as required for Exemption 4. 5 U.S.C. § 552(b)(4). Courts have held that "information is confidential for the purposes of Exemption 4 if its disclosure would have the effect either: '(1) of impairing the government's ability to obtain . . . necessary information . . . in the future, or (2) of causing substantial harm to the competitive position of the person from whom the information was obtained.'" *Inner City Press/Cmnty. on Move v. Bd. of Governors of Fed. Reserve Sys.*, 463 F.3d 239, 244 (2d Cir. 2006); *see also Nat'l Parks & Conservation Ass'n v. Morton*, 498 F.2d 765, 770 (D.C. Cir. 1974). In this case disclosure would not prevent the government from obtaining necessary information because drug manufacturers must submit trial data for approval. To the extent public disclosure reveals unfavorable information about Sarepta's practices, this is the precise type of information the government should not want to collect in the future, because drug manufacturers should not be engaging in such practices. Sarepta is also the only current successful of a potential cure for Duchenne, so disclosure would not cause substantial harm to its competitive position.

The D.C. Circuit has also twice stated that Exemption 4 requires balancing the public interest in disclosure against the interest in nondisclosure. *See Pub. Citizen Health Research Grp. v. Food & Drug Admin.*, 185 F.3d 898, 908 (D.C. Cir. 1999) (Garland, J., concurring) (citing *Washington Post Co. v. HHS,* 690 F.2d 252, 269 (D.C. Cir.1982) and *Washington Post Co. v. HHS,* 865 F.2d 320, 326-27 (D.C. Cir.1989)). In this case I have demonstrated a substantial public interest in obtaining the requested information. It is also unlikely that FDA can show that the requested information constitutes "personnel and medical files and similar files the disclosure of which would constitute a clearly unwarranted invasion of personal privacy" as required for Exemption 6. 5 U.S.C. § 552(b)(6). Furthermore, the public interest in the information regarding eteplirsen's accelerated approval outweighs any personal privacy concerns or concerns regarding trade secrets and privileged or confidential commercial or financial information.

Thank you for your consideration of this request. If you have any questions or concerns about what I am seeking, please do not hesitate to contact me at the email address below. Pursuant to the applicable FOIA provision

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and departmental regulations, I expect a response regarding this request within the ten (10) working day time limit set by law. 45 C.F.R. § 5.35(b); 5 U.S.C. § 552(a)(6)(E).

Sincerely,

s/Charles Seife

Charles Seife, Professor
ARTHUR L. CARTER JOURNALISM INSTITUTE
20 Cooper Sq, 6th Floor
New York, NY 10003
Phone: (212) 998-7970
Email: cs129@nyu.edu